

# Biostatistical Issues and the Design of Type 1 Diabetes TrialNet Protocols Agenda

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**Monday, March 7, 2005**

**8:00 a.m.–4:00 p.m.**

**Bethesda Marriott Suites, Room TBD**

**Moderators: Jim Neaton and Desmond Schatz**

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## **Introduction**

7:30 a.m. – 8:00 a.m.

**Sign-In and Light Refreshments**

8:00 a.m. – 8:15 a.m.

**Meeting Goals: What We Hope To Accomplish**

*Des Schatz*

8:15 a.m. – 8:30 a.m.

**Where We Are**

- mission of TrialNet
- number of sites
- potential patient base
- screening effort required for trials
- Natural history (description of T1DM pathogenesis) and measurement of  $\beta$ -cell function
- issues in prevention studies, early onset studies, and studies of patients with long history of T1DM
- current TrialNet studies

*Jay Skyler*

## **Design Issues in Clinical Trials**

8:30 a.m. – 9:10 a.m.

**Surrogate Endpoints: The Challenges Are Greater Than They Seem**

(10-minute discussion)

*Tom Fleming*

9:10 a.m. – 9:50 a.m.

**Some Phase 2/3 Designs**

(10-minute discussion)

*Peter Thall*

9:50 a.m. – 10:20 a.m.

**What About the Effect Size?**

(10-minute discussion)

*Jeff Mahon*

10:20 a.m. – 10:30 a.m.

**Break**

10:30 a.m. – 11:00 a.m.	<b>Discussion of Fleming, Thall, and Mahon</b> <i>Butch Tsiatis</i>
11:00 a.m. – 12:00 noon	<b>Panel Discussion</b> <i>Tom Fleming, John Lachin, Jeff Mahon, Dave Reboussin, Rich Simon, Peter Thall, Butch Tsiatis</i>
12:00 noon – 1:00 p.m.	<b>Lunch</b> ( <i>On Your Own</i> )
<b>TrialNet Design Issues</b>	
1:00 p.m. – 1:30 p.m.	<b>POPPI 1 Study as an Example</b> <i>John Lachin</i>
1:30 p.m. – 3:30 p.m.	<b>Statistical Comment</b> (15 minutes each) <i>Mark Espeland, Joan Hilton, Tom Louis,</i>
	<b>Panel and General Discussion</b> <i>Mark Espeland, Carla Greenbaum, Joan Hilton, John Lachin, Tiepu Liu, Tom Louis, Jeff Mahon, Jerry Palmer, Des Schatz, Jay Skyler</i>
<b>Summary</b>	
3:30 p.m. – 4:00 p.m.	<b>Meeting Summary</b> <i>Herman Mitchell</i>
	<b>Final Comments</b> <i>Des Schatz</i>
4:00 p.m.	<b>Adjournment</b>